## Dystrophin minigene constructs

Human dystrophin coding sequence 11058bp

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R18 R19 H3 R20 R21 R2	
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Δ4173 --N=-H1 R1 R2 R3 R22 R23 R24 H4 CR

::N:::H1 R1 R2 H3R22 R23 R24 H4 CR

Δ3990

-N=|H1| R1 | R2 | R22 | R23 | R24 | H4 | CR

Δ3849

Δ3531

--N--H1 R1 R22 R23 R24 H4 CR

-N--H1 R1 R2 R23 R24 H4 CR

Δ3510

-N-H1 R1 R2 R3 R24 H4 CR

Δ3447

-N--H1 R1 R24 H4 CR

**A2796** 

-N-|H1|R1 | H4 | CR | CT

DysM3 (3700bp)

PolyA FITR Dystrophin minigene ITR MCK Promoter

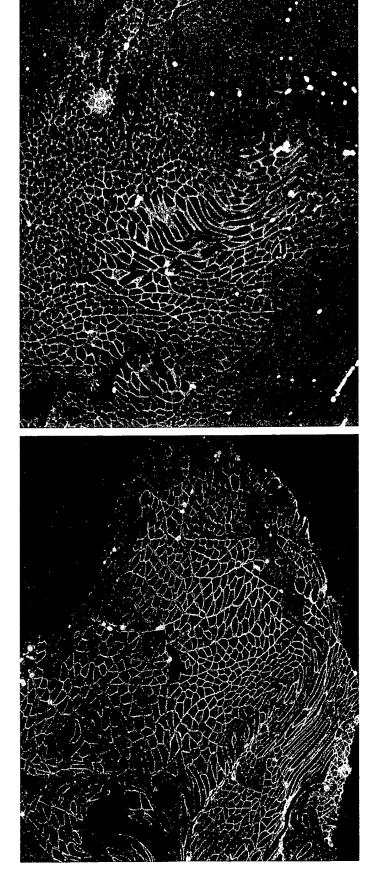
AAV-MCK-mini-dystrophin

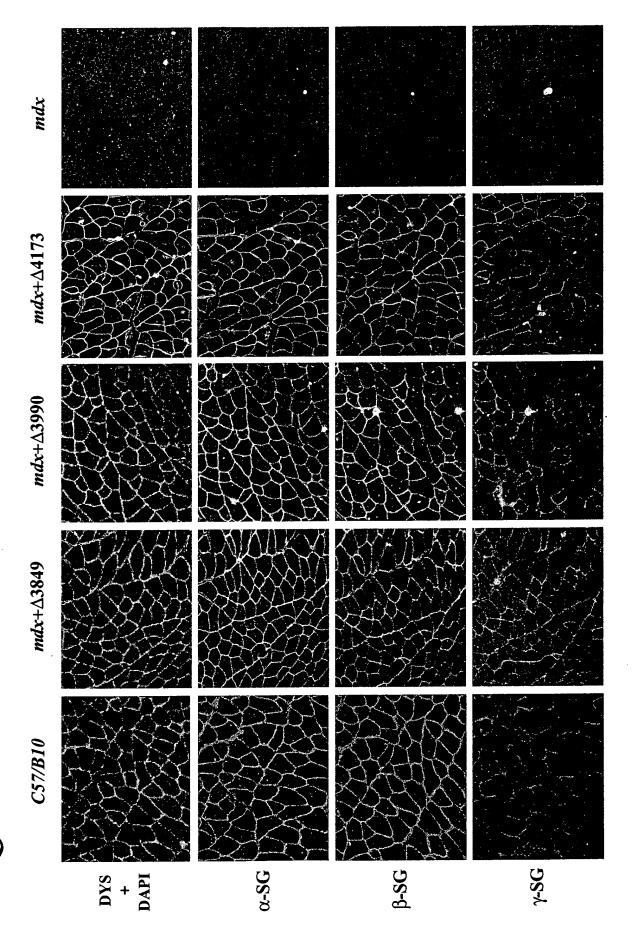
-PolyA TTR Dystrophin minigene ITR CMV Promoter

AAV-CMV-mini-dystrophin

*mdx*+∆3849

*mdx*+∆3990





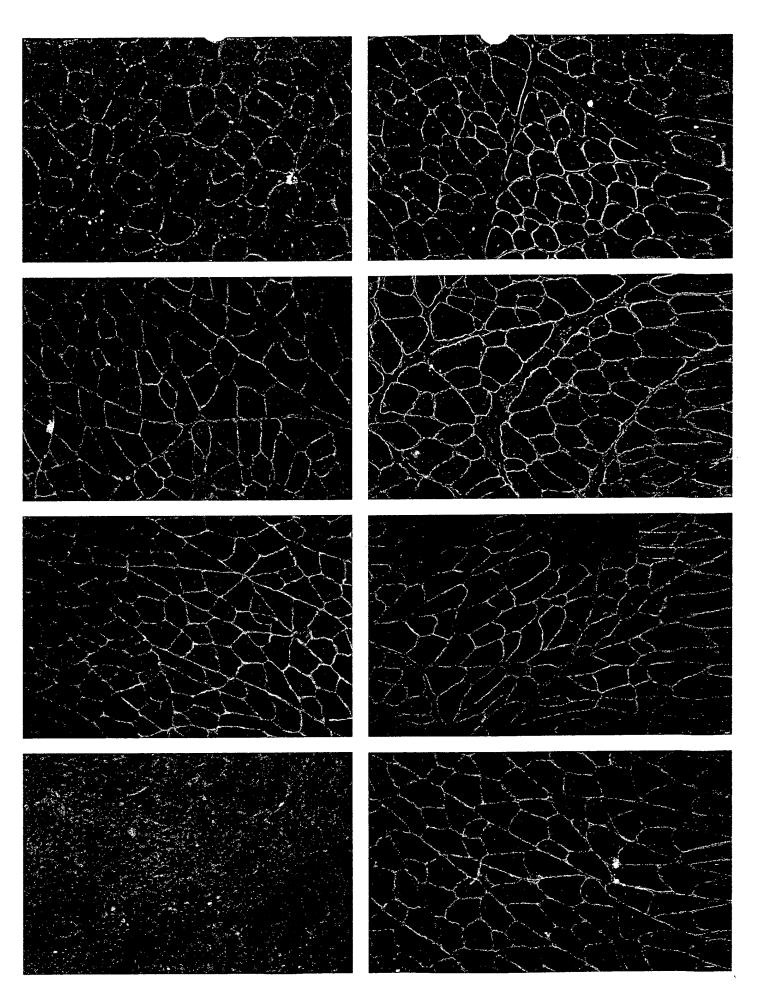


FIG. 4A

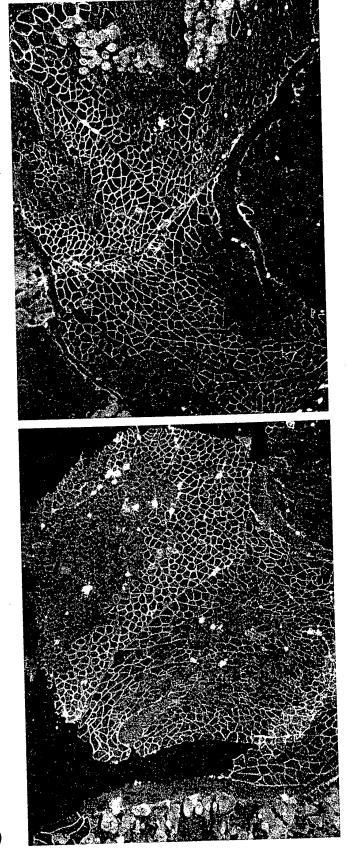


FIG. 4B

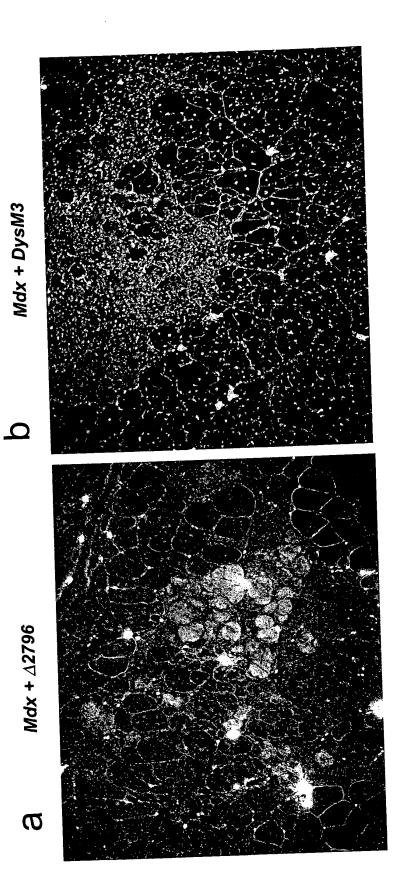


FIG. 5

Lengthening Activation

